

**RFA 10-01 CONCEPT
CIRM EARLY TRANSLATIONAL II RESEARCH AWARDS**

***PLEASE READ THE RFA WHEN IT IS RELEASED FOR FINAL AND FULL
INFORMATION***

Purpose and Objectives

The purpose of the CIRM Early Translational II Research Awards is to fund studies to move promising basic research in stem cell science toward the clinic. To this end, these awards will support two categories of research projects:

- 1) Development Candidate Awards (DC Awards) will support research that results in a novel stem cell-derived development candidate that meets an unmet medical need. By the end of the award period, these projects must have completed all necessary activities to result in a development candidate ready to start Investigational New Drug (IND)-enabling preclinical development
- 2) Development Candidate Feasibility Awards (DCF Awards) will support research directed towards the goal of obtaining a novel stem-cell-derived development candidate. Unlike the above Development Candidate Awards, by the end of the award period, applicants for these awards do not need to have completed all the necessary activities required to start preclinical development necessary for a regulatory filing. Rather, these awards are intended to allow investigators to conduct research to *identify or determine feasibility* of a given potential development candidate. For example, applicants for these awards may wish to identify a lead candidate, reproducibly establish disease-modifying activity or screen multiple candidates against a single, defined disease target.

For both types of awards, to be responsive to this RFA, applicants must have already identified a single disease target for therapeutic (diagnostic) intervention and have a scientifically justifiable hypothesis for the proposed development candidate.

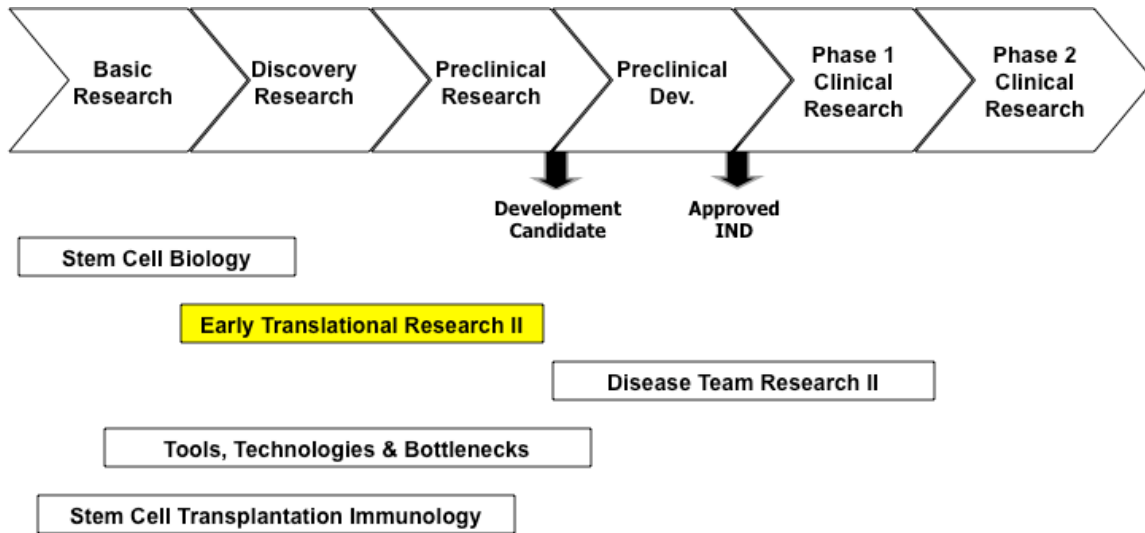
In addition, CIRM will prioritize proposals:

- Resulting in a novel cell therapy development candidate derived from pluripotent cells (DC Award);
- Leading to a novel cell therapy development candidate derived from pluripotent cells (DCF Award);
- Based on human pluripotent stem cells (e.g. human pluripotent stem cells used for drug or diagnostic discovery).
- Ineligible for or unlikely to receive timely or sufficient federal funding

Research that is outside of the scope of these awards includes:

- Target discovery.
- IND-enabling preclinical development activities (e.g. GMP production, GLP toxicology and tumorigenicity studies).
- Clinical studies. Analysis of human subject samples, if directly related to the proposed research, can be funded.

Diagram of Stage Specificity of Current and Upcoming RFAs



CIRM Award Information

CIRM proposes to fund:

- Development Candidate Awards with justifiable total requested funds for each award over three years of up to \$6.0 million for a total of up to \$60 million
- Development Candidate Feasibility Awards with justifiable total requested funds for each award over three years of up to \$2 million for a total of up to \$20 million
- Total program cost of up to \$80 million.

For-profit applicant organizations will be permitted to apply for either grants or loans.
Non-profit applicant organizations will be funded through grants

Loan Information

Loans will be administered over a six- or ten-year term to be determined by the loan applicant. Loans will be recourse or non-recourse. The interest on six-year loans will be prime plus 300 basis points; the interest on ten-year loans will be prime plus 500 basis points. The prime rate will be that on the date the ICOC authorizes funding of the loan.

CIRM Institutional Eligibility

- All CIRM supported research must be conducted in the state of California
- Open to all academic, non-profit and for-profit institutions in the state of California
- For this RFA, CIRM will use its pre-application process; therefore no limits will be applied on the number of pre-applications an eligible institution can submit.

CIRM Investigator Eligibility

As a multidisciplinary team often most effectively conducts translational research, this award will be open to:

- A Principal Investigator (PI) and, in the case of Development Candidate Awards, up to 1 Co-Principal Investigator (Co-PI) with Ph.D., M.D or equivalent degrees who are

authorized by the applicant and Co-PI institutions to conduct the proposed research in California.

- PIs and Co-PIs must commit 20% and 15% effort respectively towards programs supported under this RFA.

CIRM encourages collaborative endeavors between non-profit and for-profit institutions.

Collaborative Funding Partner Participation

CIRM has established a program with several other government agencies that fund stem cell and regenerative medicine research. Through this Collaborative Funding Partner program, California-based PIs can collaborate with a Funding Partner PI from a Funding Partner applicant institution eligible for funding from one of CIRM's collaborative funding partners to bring important additional resources to proposed projects. If a collaborative funding proposal is approved CIRM will fund all project work done within the State of California and its Funding Partner will fund all project work within its jurisdiction.

Provisional Time Table:

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| • Release of RFA 10-01 | mid Feb | 2010 |
| • Pre-Applications due | Mar 18 | 2010 |
| • Applications due | July | 2010 |
| • Grants Working Group Review of Applications | Sept | 2010 |
| • ICOC Approval | Oct | 2010 |